Stem Cell Agency Board Approves Funding for Rare Immune Disorder

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Oakland, CA – IPEX syndrome is a rare condition where the body can't control or restrain an immune response, so the person's immune cells attack their own healthy tissue. The syndrome mostly affects boys, is diagnosed in the first year of life and is often fatal. Today the governing Board of the California Institute for Regenerative Medicine (CIRM) invested almost $12 million in a therapy being tested in a clinical trial to help these patients.

Children born with IPEX syndrome have abnormalities in the FOXP3 gene. This gene controls the production of a type of immune cell called a T Regulatory or Treg cell. Without a normal FOXP3 +Treg cells other immune cells attack the body leading to the development of IPEX syndrome, Type 1 diabetes, severe eczema, damage to the small intestines and kidneys and failure to thrive.

Current treatments involve the use of steroids to suppress the immune system - which helps ease symptoms but doesn't slow down the progression of the disease - or a bone marrow stem cell transplant. However, a transplant requires a healthy, closely matched donor to reduce the risk of a potentially fatal transplant complication called graft vs host disease, in which the donated immune cells attack the recipient's tissues.

Dr. Rosa Bacchetta and her team at Stanford University have developed a therapy using the patient's own natural CD4 T cells that, in the lab, have been genetically modified to express the FoxP3 gene and converted into Treg cells. Those cells are then re-infused into the patient with a goal of determining if this approach is both safe and beneficial. Because the cells come from the patients there will be fewer concerns about the need for immunosuppressive treatment to stop the body rejecting the cells. It will also help avoid the problems of finding a healthy donor and graft vs host disease.

Dr. Bacchetta has received approval from the FDA to test this approach in a Phase 1 clinical trial for patients suffering with IPEX syndrome.

"Children with IPEX syndrome clearly represent a group of patients with an unmet medical need, and this therapy could make a huge difference in their lives," says Dr. Maria T. Millan, the President and CEO of CIRM. "Success of this treatment in this rare disease presents far-reaching potential to develop treatments for a larger number of patients with a broad array of immune disorders resulting from dysfunctional regulatory T cells."

In addition to a strong scientific recommendation to fund the project the review team also praised it for the applicants' commitment to the principles of Diversity, Equity and Inclusion in their proposal. The project proposes a wide catchment area, with a strong focus on enrolling people who are low-income, uninsured or members of traditionally overlooked racial and ethnic minority communities.

About CIRM

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and act with a sense of urgency to succeed in that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising stem cell technologies.

With $5.5 billion in funding and more than 150 active stem cell programs in our portfolio, CIRM is the world's largest institution dedicated to helping people by bringing the future of cellular medicine closer to reality.

For more information go to www.cirm.ca.gov

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